EMA/166793/2017

European Medicines Agency decision

P/0106/2017

of 11 April 2017

on the agreement of a paediatric investigation plan for human fibrinogen concentrate (BT524) (EMEA-001931-PIP01-16) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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on the agreement of a paediatric investigation plan for human fibrinogen concentrate (BT524) (EMEA-001931-PIP01-16) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Biotest AG on 10 June 2016 under Article 16(1) of Regulation (EC) No 1901/2006,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 24 February 2017, in accordance with Article 18 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the agreement of a paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for human fibrinogen concentrate (BT524), powder for solution for injection or infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

This decision is addressed to Biotest AG, Landsteinerstr. 5, D-63303 – Dreieich, Germany.

EMA/PDCO/853184/2016
London, 24 February 2017

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan

EMEA-001931-PIP01-16

Scope of the application

Active substance(s):

Human fibrinogen concentrate (BT524)

Condition(s):

Treatment of congenital fibrinogen deficiency

Pharmaceutical form(s):

Powder for solution for injection or infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Biotest AG

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Biotest AG submitted for agreement to the European Medicines Agency on 10 June 2016 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 19 July 2016.

Supplementary information was provided by the applicant on 12 December 2016. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 18 of said Regulation.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

Not applicable

2. Paediatric investigation plan

2.1. Condition

Treatment of congenital fibrinogen deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of congenital fibrinogen deficiency

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder for solution for injection or infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	1	**Study 1** Open-label, non-controlled PK/PD and safety and efficacy study: Part I: investigation of 14 day single-dose pharmacokinetics and pharmacodynamics of human fibrinogen concentrate in patients from birth to less than 18 years of age (and adults) with congenital fibrinogen deficiency (afibrinogenemia or severe hypofibrinogenemia). Part II: investigation of hemostatic efficacy and safety of single and/or repetitive intravenous infusions of human fibrinogen concentrate for on-demand prophylaxis and/or on-demand treatment of bleeding events in patients from birth to less than 18 years of age (and adults) with congenital fibrinogen deficiency (afibrinogenemia or severe hypofibrinogenemia) (Study 984).

Extrapolation, modelling and simulation studies	2	**Study 2** Semi-mechanistic population PK/PD model (Fibrinogen Antigen/Fibrinogen Activity) with subsequent link to the surrogate of efficacy (maximum clot firmness) to support the dosing strategy and activity of human fibrinogen concentrate in patients from birth to less than 18 years of age with congenital fibrinogen deficiency (afibrinogenemia or severe hypofibrinogenemia). **Study 3** Extrapolation study to support the understanding of dosing and efficacy of human fibrinogen concentrate in patients from birth to less than 18 years of age with congenital fibrinogen deficiency (afibrinogenemia or severe hypofibrinogenemia) using data obtained in part I and II of Study 984 (PIP study 1).
Other studies	0	Not applicable
Other measures	0	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2018
Deferral for one or more measures contained in the paediatric investigation plan:	No